# Non-interventional study for long term documentation of treatment with Vihuma in patients with hemophilia A (Biotest NIS-019)

**First published:** 26/04/2017

**Last updated:** 02/07/2024

Study Finalised

## Administrative details

**EU PAS number**

EUPAS18757

**Study ID**

33454

**DARWIN EU® study**

No

**Study countries**

- ☐ Austria
- ☐ Germany
- ☐ Switzerland

**Study description**

The non-interventional Study has been cancelled due to the lack of participating sites and patient numbers at this time. No sites or patients have been recruited.Vihuma is a recombinant factor VIII (FVIII) produced in the human cell line HEK 293F (Human Embryonic Kidney Cells). Vihuma is approved for treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency). Vihuma can be used for all age groups. Details are given in the SPC and German Fachinformation of Vihuma.Hemophilia A is an inherited, chronic bleeding disorder and patients have to be treated lifelong with FVIII concentrates. Most children and adolescents are treated prophylactically in industrialized countries. Prophylaxis has the goal to avoid bleedings, in order to guarantee the patient a high quality of life (QoL).This will be the first NIS allowing direct comparison of treatment with a recombinant (Vihuma) and plasmatic factor VIII product (Haemoctin SDH, Biotest NIS-016), since data obtained in this two NIS are very similar.

---

**Study status**

Finalised

# Research institutions and networks

## Institutions

### Biotest

**First published:** 01/02/2024

**Last updated:** 01/02/2024

**Institution**

Multiple centres: 18 centres are involved in the study

## Contact details

**Study institution contact**

Artur Bauhofer artur.bauhofer@biotest.com

Study contact

artur.bauhofer@biotest.com

**Primary lead investigator**

Wolfgang Miesbach

Primary lead investigator

## Study timelines

**Date when funding contract was signed**

Planned: 23/02/2017

Actual: 02/03/2017

---

**Study start date**

Planned: 02/01/2020

Actual: 31/12/2019

---

**Data analysis start date**

Planned: 30/06/2025

Actual: 31/12/2019

**Date of final study report**

Planned: 31/12/2025

Actual: 31/12/2019

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Biotest AG

## Study protocol

2017-04-12 FVIII recombinant NIS Observation Plan 1.0.pdf (215.44 KB)

## Regulatory

**Was the study required by a regulatory body?**

No

**Is the study required by a Risk Management Plan (RMP)?**

Not applicable

# Methodological aspects

## Study type

## Study type list

**Study topic:**

Disease /health condition

Human medicinal product

---

**Study type:**

Non-interventional study

---

**Scope of the study:**

Drug utilisation

Effectiveness study (incl. comparative)

**Data collection methods:**

Primary data collection

---

**Main study objective:**

With this NIS long-term data for the effectiveness in bleeding prevention and on QoL will be generated.What is the dose and frequency of Vihuma in prophylaxis?Is it possible to reduce the dose or extend the frequency of applications compared to previously used factor VIII products?What are the factors influencing the risk of bleeding over the time of treatment?

## Study Design

**Non-interventional study design**

Other

---

**Non-interventional study design, other**

Prospective, single arm study

## Study drug and medical condition

**Medicinal product name**

VIHUMA

**Medical condition to be studied**

Factor VIII deficiency

# Population studied

**Short description of the study population**

Patients with hemophilia A treated with Vihuma recombinant factor VIII (FVIII) at home and, in some exceptional cases, in the clinic or a local doctor's practice.
Patients with following criteria were included:
1. Treatment in accordance with the SPC for Vihuma
2. Children of all ages and adult patients with FVIII deficiency (previously treated and previously untreated patients)
3. Written informed consent to allow data collection and data transfer to third party

**Age groups**

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)
- Infants and toddlers (28 days – 23 months)
- Term newborn infants (0 – 27 days)
- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)
- Adults (85 years and over)

**Special population of interest**

Immunocompromised

**Estimated number of subjects**

60

## Study design details

**Outcomes**

Annual bleeding rate defined as episodes per year in patients with Vihuma treatment, differentiated by prophylaxis and on demand treatmentDose and frequency of Vihuma applications in comparison to previously used factor VIII products, AE and subsequent suspected ADR (AE assessed as causally related with Vihuma treatment)AE with bleeding = AE of special interest (AESI) with extended bleeding documentation, for e.g. if the duration and severity of the bleeding is within the situation as expected or unexpectedOccurrence and characterization of FVIII inhibitors to Vihuma

**Data analysis plan**

All analyses will be performed in an exploratory sense. Data will be analyzed using descriptive statistics. For continuous variables, mean, standard deviation, minimum, maximum, median, 25% and 75% percentiles will be presented. Qualitative and categorical variables will be presented by means of absolute and relative frequencies. A medical evaluation of the findings will be performed. Details of analysis will be described in a statistical analysis plan.

## Data management

## ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

**Data sources (types)**

Other

**Data sources (types), other**

Prospective patient-based data collection

## Use of a Common Data Model (CDM)

**CDM mapping**

No

## Data quality specifications

**Check conformance**

Unknown

**Check completeness**

Unknown

**Check stability**

Unknown

**Check logical consistency**

Unknown

# Data characterisation

**Data characterisation conducted**

No